Application Number: T1-00005 Scientific Score: 94

Title: California Institute for Regenerative Medicine (CIRM) Type I Comprehensive Training Program

Specific names of individuals and institutions are blacked out to preserve applicant confidentiality where possible.

Proposal Abstract as Submitted by Applicant

The aim of the Type1, Comprehensive Training Program is to train basic
scientists, engineers and physicians to become leaders in stem cell research and clinical
programs in academia and industry. A distinctive feature of the program is that
Scholars will be trained from a multidisciplinary perspective, which is possible because
faculty from the College of Letters and Science, and the Schools of Dentistry,
Engineering, Law, Nursing, Medicine, and Public Affairs are located in close proximity
on the same campus and have developed a tradition of multi-disciplinary teaching and
research collaboration. The recently established Institute for Stem Cell Biology
and Medicine (ISCBM) has been built on this foundation and has received strong
campus-wide support as evidenced by the allocation of 12 new faculty positions in stem
cell biology and a major space commitment. The ISCBM will coordinate the training of 5
pre-doctoral, 5 post-doctoral, and 6 clinical Scholars, each of whom will be presented
with numerous training options. Some may choose to work with faculty who are
leaders in cell and molecular biology, while others will elect to receive training in gene
medicine, cell-based therapies, and organ transplantation from clinician-scientists already
applying these new procedures to patient care. As part of this training Scholars will
develop an understanding of Good Manufacturing Policies (GMP) and compliance issues
related to clinical trials. Scholars with interests in bioengineering and nanotechnology can
work with faculty developing methods for manipulating and visualizing cells using
microfluidic systems, bio-mechanical tools, and nano-scale microscopes or whole body
imagers. The training program will also accommodate Scholars interested in social, legal,
or policy aspects of stem cell research. In addition to their individualized research
program, all Scholars will participate in numerous common activities, including a new
course in Stem Cell Biology that includes lectures and discussion sessions on adult and
embryonic stem cells (ESC), organogenesis, tissue repair, nuclear reprogramming, gene
therapy, the conduct of clinical trials, and social, legal, and ethical aspects of stem cells.
Scholars will also have the opportunity to join one or more journal clubs, attend research
seminars featuring leading stem cell biologists, and present talks describing their own
research. Regardless of their particular interest, by the end of the training, Clinical
Scholars, many of whom are simultaneously working for a graduate degree and board
certification through the will obtain an appreciation of basic research and basic
scientists and engineers will acquire knowledge human disease and an understanding of
how research advances are translated to the clinic. Whether they ultimately work
individually or as part of a multidisciplinary team, the program will train highly
skilled scientists and clinicians who will help to make the practice of regenerative
medicine a reality.

This program will benefit the people and the state of California by providing high-quality training in the scientific, clinical, social, and ethical aspects of stem cell research to the scientists and clinicians who will develop and apply future therapies in this rapidly emerging field.

Summary of Review

This application proposes a multidisciplinary type I program to train basic scientists, engineers, and physicians in stem cell research and related clinical aspects. The program builds on a recently established institute for stem cell biology within this institution and draws on faculty from multiple departments, including those in the schools of dentistry, nursing, engineering, law, medicine, and public affairs. The new institute allocates 12 new faculty positions and provides a major space commitment for stem cell research. The program will be led jointly by two program directors, both with outstanding records as scientists and as administrators. One director, who is a member of the National Academies of Science, has given up all other administrative duties to run the new institute and this training program. A steering committee composed of the co-directors and two additional faculty members with a background in neural stem cell research will oversee all program activities. Appropriate mechanisms for trainee mentoring and evaluation, and program evaluation are in place. Of note are the large and talented pool of applicants and the strength of the current training program in clinical research from which clinical CIRM trainees will be drawn. Proposed mentors number 60, many of whom are already working with mouse or human embryonic stem/progenitor cells. The faculty has a very strong record of mentoring, of external funding, and a number of wellestablished training grants. The required courses are integrated into one course of parallel didactic lectures and discussion with papers reviewed. The institution has an effective outreach program for recruiting underrepresented minorities into science and engineering. Overall, this proposal presents a comprehensive near complete and exciting stem cell biology-training program.

Overall Strengths and Weaknesses

This comprehensive program has much notable strength and few weaknesses. It builds on a strong foundation of a newly formed stem cell institute, strong pre-existing interest in stem cells and their medical use, and a strong research community of varied interests and expertise. The required course and assessment of the trainees are well delineated and well thought out. Weaknesses are mostly related to the formal aspects of the application, like the absence of information on previous training programs.

Recommendations

Highly meritorious and recommended for funding.

	Pre	Post	Clinical	Total
Fellows Requested:	5	5	6	16
Fellows Recommended:	5	5	6	16

	Year 1	Total
Budget Requested:	\$ 1,250,000	\$ 3,750,000
Budget Recommended:	\$ 1,250,000	\$ 3,750,000